

**ROUTING SLIP
GENERATED BY: HF-40
DATE: JUL 14, 2004**

FDA CONTROL NUMBER: 04 3877

TRACER #: **OS #:**

DATE OF CORRESPONDENCE: 07/06/04

DATE INTO FDA: 07/14/04

TO: LESTER M CRAWFORD, ACTING COMMISSIONER OF THE FOOD AND DRUG
ADMINISTRATION

FROM: DAYTON T REARDAN, ORPHAN MEDICAL, INC.

SYNOPSIS: FORWARDS COPY OF LETTER FROM ABBEY MEYERS (NATIONAL ORGANIZATION
FOR RARE DISORDERS, INC.). REQUESTS THAT LETTER BE ADDED TO
PENDING CITIZEN PETITION (DOCKET # 003P-0039/CPI).

LEAD OFFICE: HFA-305

HOME OFFICE: HF-40

CONTACT/PHONE#: CAPRI R MCCLENDON 301-827-5903

COPIES: HF-40 ELIZABETH A CLARKE

COORDINATION:

SIGNATURE REQUIRED:

REFERRALS FROM HF-40

ASSIGNED TO	ACTION	DUE DATE
----- HFA-305	----- NECESSARY ACTION	-----



July 6, 2004

Lester M. Crawford, DVM, PhD
Acting Commissioner [HF-2]
Food and Drug Administration
Room 14-71
Parklawn Building,
5600 Fishers Lane
Rockville, MD 20857
Phone: 301-827-2410

**Subject: Docket Number 03P-0039/CP1
Request Addition of NORD Letter to Current Citizen Petition**

Dear Dr. Crawford:

On January 28, 2003, our Company submitted a Citizen Petition to address serious issues that have arisen in the application of product and establishment user fees to orphan drugs. On April 22, 2003, Orphan Medical met with Dan Troy and other FDA staff regarding this Citizen Petition.

We respectfully request you add the attached May 7, 2004 letter authored by Abbey Meyers, President of the National Organization for Rare Disorders, Inc. to this pending, yet to be resolved, Citizen's Petition docket.

Please contact me directly, should you have any questions or concerns regarding this correspondence.

Sincerely yours,

A handwritten signature in black ink, appearing to read "Dayton T. Reardan".

Dayton T. Reardan, PhD, RAC
Vice President of Regulatory Affairs
Orphan Medical, Inc.
Direct: 952-513-6969

Enclosure

cc: Abbey Meyers, NORD
Jane Axelrad, FDA – CDER
John McCormick, FDA – OOPD
Bo Allen, Rare Disease Therapeutics

R:\FDA\CORRE\Citizen_Petition\Add Nord Letter 6-Jul-04\Cover Letter 6-Jul-04.doc

1

cl 3877

National Organization for Rare Disorders, Inc.®

NORD • 55 Kenosia Avenue, P.O. Box 1968 • Danbury, CT 06813-1968

Tel: 203-744-0100 • FAX: 203-798-2291

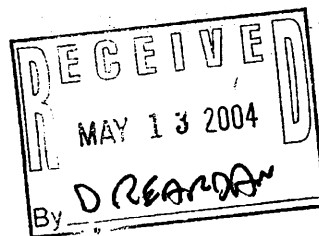
TDD (for hearing impaired) (203) 797-9590

<http://www.rarediseases.org> • e-mail: orphan@rarediseases.org



May 7, 2004

Marlene E. Haffner, M.D.
FDA Office For Orphan Products
HF35, RM 8-73
5600 Fishers Lane
Rockville, MD 20857



Dear Marlene:

In response to Bo Allen's letter of May 3, I want to say on behalf of rare disease patients that Bo is absolutely correct; something must be done about the ludicrous user fees that FDA is charging to manufacturers of orphan products.

As you know, I attended a meeting last year with Dan Troy and Orphan Medical (Jack McCormack was there) about this same topic. In that situation, Orphan Medical had a product with one-half million dollars annual sales, and the FDA demanded user fees totaling more than a half million dollars. In that case, the woman in charge of user fees said the manufacturer has to be a small company with less than \$10 million in sales; therefore, despite the ridiculous situation with this particular half million-dollar drug, Orphan Medical was not eligible for the waiver.

As you know, it was NORD's advocacy efforts that got the orphan drug user fee waiver inserted in one of the PDUFA laws. Since FDA refuses to budge on this issue, the agency will leave us no other option but to develop another amendment that will force the agency to observe the intent of the waiver. It is ludicrous that FDA uses a definition for a small business that is different than all other government agencies. I believe the definition used by the agency is a business with less than \$10 million annual sales, whereas the small business administration's definition is much more liberal.

In the case of Rare Disease Therapeutics (RDT), the FDA is looking at the sales of Swedish Orphan and not the sales of RDT. RDT licenses the drug from Swedish Orphan so only RDT's annual revenues should be recognized. Similarly, FDA counts the sales of all of Orphan Medical's drugs inside and outside of the USA, and that should not be allowed because the sales of all products the company makes and sells in all countries is not related to the user fees for one orphan drug sold to a small number of people in the USA.

NATIONAL MEMBER ORGANIZATIONS

Alagille Syndrome Alliance
Alpha 1 Association
Alpha 1 Foundation
American Brain Tumor Association
American Laryngeal Papilloma Foundation
American Porphyria Foundation
American Syringomyelia Alliance Project
Aplastic Anemia & MDS International Foundation, Inc.
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc. (AGMD)
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation
Charcot-Marie Tooth Association
Chromosome 18 Registry Research Society
Cleft Palate Foundation
Cornelia De Lange Syndrome Foundation
Cystinosis Foundation, Inc.
DEBRA of America
Dysautonomia Foundation, Inc.
Dystonia Medical Research Foundation
Ehlers Danlos National Foundation
Epilepsy Foundation
Families of Spinal Muscular Atrophy
Foundation for Ichthyosis and Related Skin Types
Genetic Alliance
Guillain Barre Syndrome Foundation International
Hemochromatosis Foundation
Hereditary Colon Cancer Association
Hereditary Disease Foundation
HHT Foundation International, Inc.
Histiocytosis Association of America
Huntington's Disease Society of America
Immune Deficiency Foundation
International FOP Association, Inc.
International Joseph Diseases Foundation, Inc.
International Rett Syndrome Association
Interstitial Cystitis Association
Lowe Syndrome Association, Inc.
Mastocytosis Society, Inc.
Mucopolidosis Type IV Foundation, Inc.
Myasthenia Gravis Foundation of America, Inc.
Myeloproliferative Disease Research Center
Ostitis Association of America, Inc.
Osteoporosis Network, Inc.
National Adrenal Disease Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Foundation for Ectodermal Dysplasias
National Hemophilia Foundation
National Marfan Foundation
National MPS Society, Inc.
National Multiple Sclerosis Society
National Neurofibromatosis Foundation
National PKU News
National Spasmodic Torticollis Association
National Tay Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
Neurofibromatosis, Inc.
Osteogenesis Imperfecta Foundation
Parkinson's Disease Foundation, Inc.
Platelet Disorder Support Association
Prader Willi Syndrome Association, USA
Pulmonary Hypertension Association
PXE International, Inc.
Reflex Sympathetic Dystrophy Syndrome Association
Scleroderma Foundation
Sickle Cell Disease Association of America
Stevens Johnson Syndrome Foundation
Sturge-Weber Foundation
The Erythromelalgia Association
The Oxalosis and Hyperoxaluria Foundation
The Paget Foundation
Tourette Syndrome Association
Trigeminal Neuralgia Association
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
VHL Family Alliance
Wegener's Granulomatosis Association
Williams Syndrome Association
Wilson's Disease Association

Associate Member Organizations

Acid Maltase Deficiency Association (AMDA)
American Autoimmune Related Disease Association
American Behcet's Disease Association
Acid Self-Help Group Clearinghouse
Trophic Lateral Sclerosis (ALS) of Greater Philadelphia Chapter
Association for People with the Van Lohuizen Syndrome (CMTC)
A-T Children's Project
(The) CDG Family Network Foundation

Canadian Organization for Rare Disorders (CORD)
Children's PKU Network
Chromosome Deletion Outreach Inc.
Chronic Granulomatous Disease Association
CLIMB
Consortium of Multiple Sclerosis Centers
Contact A Family
Cushing Support & Research Foundation, Inc.
EURORDIS
Family Caregiver Alliance

Family Support Network of North Carolina
Freeman-Sheldon Parent Support Group
Hydrocephalus Association
Incontinentia Pigmenti International Foundation
K-T Support Group
Late Onset Tay-Sachs Foundation
Les Turner ALS Foundation, Ltd.
Mercy Medical Airlift
National Lymphedema Network, Inc.
National Niemann-Pick Disease Foundation

National Spasmodic Dysphonia Association
Organic Acidemia Association
Osteoporosis and Related Bone Diseases National Resource Center
Parent to Parent New Zealand, Inc.
Rare & Expensive Disease Management Program (REM)
Recurrent Respiratory Papillomatosis Foundation
Restless Legs Syndrome Foundation
Sarcoid Networking Association

Shwachman - Diamond Syndrome International
Society for Progressive Supranuclear Palsy, Inc.
Sotos Syndrome Support Association
Takayasu's Arteritis Association
Taiwan Foundation for Rare Disorders

Associations are joining continuously. For newest listing, please contact the NORD office.

Dedicated to Helping People with Orphan Diseases

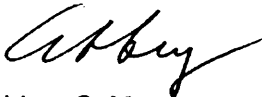
Marlene E. Haffner, M.D.
May 7, 2004
Page Two

The bottom line is that patients are paying a huge unbearable tax on orphan drugs. Companies must pass these expenses onto patients, and the rarer a disease is the higher the tax is. As Bo points out, patients with common conditions pay just a few pennies compared to thousands of dollars tacked on to each orphan drug.

Marlene, I hope you can do something to find an amicable solution because it is prejudicial to allow this situation to continue. As patient advocates, we must do something to stop these costs from being passed onto patients. The amendment that authorized the waiver makes it clear that FDA cannot expect rare disease patients to bear these costs, whereas if you increase user fees for a drug like Celebrex or Viagra, it would hardly be noticeable (amounting to fractions of pennies per person). If we must resort to the legislative route, FDA may not like the language of a new amendment so it would be much better to resolve this user fee problem amicably.

I look forward to your reply.

Very truly yours,



Abbey S. Meyers
President

ASM:aa

cc: Bo Allen
Milton Ellis
Diane Dorman
Dr. Jack McCormack

bc: Dayton Reardon
John Bullion